



## **Amicus Therapeutics Presents Positive Data Update From Phase 2 Extension Study of Amigal(TM) for Fabry Disease**

### **Company also presents encouraging preclinical data for Chaperone and ERT Combination Therapy and Parkinson's disease programs**

CRANBURY, N.J., Feb 11, 2010 /PRNewswire via COMTEX News Network/ -- Amicus Therapeutics (Nasdaq: FOLD) announced today additional positive preliminary data from its ongoing Phase 2 extension study of its investigational drug Amigal(TM) (migalastat HCl) for Fabry disease at the Lysosomal Disease Network WORLD Symposium in Miami, Florida. The Company also presented encouraging data from preclinical studies evaluating the combination of pharmacological chaperones and enzyme replacement therapy (ERT) for Fabry disease and Pompe disease as well as from preclinical studies examining the use of pharmacological chaperones for the treatment of Parkinson's disease.

#### **Phase 2 Extension Study Overview**

Twenty-six subjects completed either 12 or 24 weeks of treatment with migalastat HCl during the initial Phase 2 studies and twenty-three subjects enrolled in a separate, voluntary long-term extension study designed to evaluate the long-term safety and efficacy of migalastat HCl. Over the course of the initial Phase 2 and extension studies, fifteen subjects have been treated with migalastat HCl for approximately 2-3 years and eight subjects have been treated with migalastat HCl for more than 3 years. Twenty-one subjects continue to receive treatment in the ongoing extension study.

#### **Preliminary Data Update from Long-term Phase 2 Extension Study**

During the course of the extension study, treatment with migalastat HCl has been generally well-tolerated, with no drug-related serious adverse events. The most common adverse events were headache, arthralgia and diarrhea.

Renal function is being evaluated by two measures in the extension study, estimated glomerular filtration rate (eGFR) and proteinuria. Preliminary data indicate that eGFR has remained stable out to 2-3 years for all subjects continuing in the extension study and the average annual rate of change in eGFR in subjects identified as responders to migalastat HCl, excluding hyperfiltrators, was +2.0 mL/min/1.73m<sup>2</sup>. Additionally, trends of reduced proteinuria continued to be observed in subjects identified as responders to migalastat HCl.

Derralynn Hughes, MA, DPhil, FRCPATH, Senior Lecturer in Haematology Department Academic Haematology, Royal Free & University College Medical School, London, UK, stated, "The additional renal function data with migalastat from the ongoing extension study are encouraging. The eGFR results were particularly of interest as the data at this point in the study compare favorably to the previously published eGFR literature in untreated and ERT-treated Fabry patients."

As previously announced, a Phase 3 study intended to support approval in the United States (Study 011) commenced in the second quarter of 2009 and treatment of the first patient began in the fourth quarter of 2009. The Company expects to complete enrollment by the end of 2010 and to have preliminary results in mid-2011.

In addition, the Company expects to commence a separate Phase 3 study (Study 012) before year end. Study 012 is intended to support approval in the European Union and will be an 18-month, randomized, open-label study comparing migalastat HCl to ERT in approximately 60 subjects. The primary outcome of efficacy will be renal function as measured by glomerular filtration rate (GFR).

John F. Crowley, Chairman and CEO of Amicus Therapeutics, added, "We are very pleased with the continued positive Amigal data and remain confident that our Phase 3 program will be successful. Our focus is the completion of enrollment in Study 011 this year and the commencement of Study 012. We are also very encouraged by the results of our preclinical studies of the chaperone-ERT combination approach as well as the advancements with our Parkinson's program. We look forward to moving these applications of our technology forward in 2010."

#### **Preclinical Chaperone-ERT Combination Therapy Data**

Today Amicus presented data from preclinical studies that evaluated the combination of migalastat HCl and ERT and AT2220

and ERT in mouse models of Fabry and Pompe disease, respectively. Studies of both combinations demonstrated that co-administration of the chaperone with ERT resulted in prolonged half-life of ERT in the circulation, increased enzyme activity in cells and greater substrate reduction in target tissues compared to that seen with ERT alone.

### **Preclinical Parkinson's Disease Data**

Amicus also presented data from preclinical studies that evaluated the chaperone AT2101 in mouse models of Parkinson's disease. The studies demonstrated that treatment with AT2101 increased the activity of beta-glucocerebrosidase (GCase), prevented accumulation of alpha-synuclein in the brain and improved motor function as assessed in various behavioral tests. The Company also reported that new compounds have been identified that improve on the properties of AT2101 and expand the range of doses and regimens that show motor improvement in mouse models of the disease.

### **About Fabry Disease**

Fabry disease is a lysosomal storage disorder caused by inherited genetic mutations in the *GLA* gene, which results in deficient activity of the enzyme alpha-galactosidase A (alpha-Gal A). Deficient alpha-Gal A activity leads to lysosomal accumulation of globotriaosylceramide (GL-3), which is believed to cause the various symptoms of Fabry disease, including pain, kidney failure and increased risk of heart attack and stroke. Migalstat HCl is designed to selectively bind to and stabilize alpha-Gal A, which facilitates proper trafficking of the enzyme to the lysosomes, where it is needed to break down GL-3.

Fabry disease is estimated to affect approximately 5,000 to 10,000 people in the developed world, but recent evidence suggests that the disease may be significantly under-diagnosed. The U.S. Food and Drug Administration's Office of Orphan Products Development has granted orphan designation for Amigal in the United States, and the European Commission has designated Amigal as an orphan medicinal product in the European Union.

### **About Pompe Disease**

Pompe disease affects an estimated 5,000-10,000 individuals world-wide and is clinically heterogeneous in the age of onset, the extent of organ involvement, and the rate of progression. The early onset form of the disease is the most severe, progresses most rapidly, and is characterized by musculoskeletal, pulmonary, gastrointestinal, and cardiac symptoms that usually lead to death from cardio-respiratory failure between 1 and 2 years of age. The late onset form of the disease begins between childhood and adulthood and has a slower rate of progression that is characterized by musculoskeletal and pulmonary symptoms that usually lead to progressive muscle weakness and respiratory insufficiency. A high majority of patients have the late onset form of the disease. The U.S. Food and Drug Administration's Office of Orphan Products Development has granted orphan drug designation for the active ingredient in AT2220 in the United States.

### **About Parkinson's Disease**

Parkinson's disease (PD) affects more than one million people in the United States alone. Age is the greatest risk factor for development of PD, with a prevalence of about 1% at age 65 that increases with advancing age. Genetic risk factors have also been identified. Recent studies have shown that mutations in the *Gba* gene for the lysosomal enzyme acid beta-glucocerebrosidase (GCase) are the most common genetic risk factor for PD identified to date. These studies indicate that Parkinson's patients are five-fold more likely than healthy individuals to be Gaucher carriers, meaning that they have one mutant *Gba* gene on one of their two copies of chromosome 1.

### **About Amicus Therapeutics**

Amicus Therapeutics is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and diseases of neurodegeneration. Amicus' lead program is in Phase 3 for the treatment of Fabry disease.

### **Forward Looking Statements**

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of Amicus' candidate drug products, and the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "plan," "targets," "likely," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the goals, progress, timing and outcomes of ongoing discussions with regulatory authorities and the potential goals, progress,

timing and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that the company will need additional funding to complete all of its studies and, our dependence on third parties in the conduct of our clinical studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Additionally, all forward looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2008, and our other public filings with the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, and Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

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